

WHAT IS CLAIMED IS:

- 1 1. A method of performing *ex vivo* expansion of a gene-modified hematopoietic
2 stem cell comprising:
 - 3 (a) transducing a hematopoietic stem cell with a nucleic acid
4 encoding an ABC transporter; wherein the hematopoietic stem cell is transduced to
5 become a gene-modified hematopoietic stem cell; and
 - 6 (b) culturing the gene-modified hematopoietic stem cell *ex vivo*
7 wherein the gene-modified hematopoietic stem cell is expanded.
- 1 2. The method of Claim 1 wherein the ABC transporter is selected from the
2 group consisting of MDR1 and BCRP.
- 1 3. The method of Claim 1 wherein said culturing is performed in the presence of
2 an early-acting hematopoietic cytokine.
- 1 4. The method of Claim 1 wherein the cytokine is selected from the group of
2 cytokines consisting of interleukin-3, interleukin-6, G-CSF, GM-CSF, FLT-3 ligand,
3 and stem cell factor.
- 1 5. The method of Claim 1 wherein transducing the hematopoietic stem cell with
2 a nucleic acid encoding an ABC transporter is performed with a viral vector
3 comprising a nucleic acid encoding the ABC transporter.
- 1 6. The method of Claim 5 wherein the viral vector is selected from the group of
2 viral vectors consisting of a herpes simplex viral vector, an adenoviral vector, and
3 adeno-associated viral vector (AAV).
- 1 7. The method of Claim 5 wherein the viral vector is a retroviral vector.
- 1 8. The method of Claim 7 wherein the retroviral vector is a Harvey Murine
2 Sarcoma Vector and the hematopoietic stem cell is transduced by co-culture on
3 retroviral producer cell lines.

1 9. The method of Claim 1 wherein transducing the hematopoietic stem cell with
2 a nucleic acid encoding ABC transporter is performed with a DNA vector comprising
3 a nucleic acid encoding the ABC transporter.

1 10. The method of Claim 1 wherein the hematopoietic stem cell is a mammalian
2 hematopoietic stem cell.

1 11. The method of Claim 9 wherein the gene-modified hematopoietic stem cell
2 expresses a splice-corrected version of the human MDR1.

1 12. The method of Claim 9 wherein the mammalian hematopoietic stem cell is a
2 murine hematopoietic stem cell.

1 13. The method of Claim 9 wherein the mammalian hematopoietic stem cell is a
2 human hematopoietic stem cell.

1 14. A gene-modified mammalian hematopoietic stem cell that has been (i)
2 transduced with a nucleic acid encoding an ABC transporter selected from the group
3 consisting of MDR1 and BCRP, and (ii) expanded.

1 15. The gene-modified hematopoietic stem cell of Claim 14 that has been
2 expanded for at least 9 days.

1 16. A method of engrafting a mammal with the gene-modified hematopoietic stem
2 cell of Claim 15 comprising placing said cell into the mammal.

1 17. The method of Claim 16 wherein the gene-modified hematopoietic stem cell
2 further comprises a heterologous gene, and wherein the expression of the
3 heterologous gene by the gene-modified hematopoietic stem cell in the mammal aids
4 in the treatment of a disease associated with a dysfunctional cell; wherein said
5 dysfunctional cell is derived from an hematopoietic stem cell.

- 1 18. The method of Claim 16 wherein said placing is performed by injecting.
- 1 19. The method of Claim 18 wherein said injecting is repeated more than once.
- 1 20. The method of Claim 16 wherein said engrafting is stable for at least six
2 months.
- 1 21. The method of Claim 16 wherein said mammal is a human.
- 1 22. A method of identifying a stem cell comprising:
2 (a) obtaining a cell sample which contains a stem cell; and
3 (b) detecting the expression of BCRP in the cell sample; wherein a stem
4 cell is identified in the cell sample if BCRP is expressed.
- 1 23. The method of Claim 22 wherein said detecting is performed with an antibody
2 that binds to BCRP and wherein a stem cell is identified if it binds to said antibody.
- 1 24. A method of isolating a stem cell comprising:
2 (a) obtaining a cell sample which contains a stem cell;
3 (b) contacting the cell sample with an antibody that binds to BCRP; and
4 (c) isolating a cell from the cell sample that binds to said antibody;
5 wherein the isolated cell is a stem cell.
- 1 25. The method of Claim 24 wherein said isolating is performed by flow
2 cytometry.
- 1 26. The method of Claim 25, wherein said antibody has a fluorescent label and
2 said isolating is performed by fluorescence-activated cell sorting.

- 1 27. A method of diagnosing acute myelogenous leukemia (AML) in a human
2 subject comprising:
3 (a) obtaining a leukemic cell from the subject; and
4 (b) determining whether BCRP is overexpressed in the leukemic cell;
5 wherein a patient is diagnosed as having AML when BCRP is overexpressed in the
6 leukemic cell.
- 1 28. The method of Claim 27, wherein the leukemic cell is a blast cell.

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